Press Release

Rilzabrutinib granted orphan drug designation in the US for two rare diseases with no approved medicines

- Designation granted for warm autoimmune hemolytic anemia and IgG4-related disease
- Rilzabrutinib is currently under regulatory review in the US, EU and China for potential use in immune thrombocytopenia

Paris, April 3, 2025. The US Food and Drug Administration (FDA) has granted orphan drug designation to rilzabrutinib, an investigational, novel, advanced, oral, reversible Bruton's tyrosine kinase (BTK) inhibitor, for two rare diseases, warm autoimmune hemolytic anemia (wAIHA) and IgG4-related disease (IgG4-RD). There is still a significant unmet medical need for these two rare diseases, and neither have any currently approved medicine. FDA grants orphan drug designation to investigational therapies addressing rare medical diseases or conditions that affect fewer than 200,000 people in the US.

> ***Karin Knobe, MD, PhD***
> Global Head of Development, Rare Diseases
> *"Orphan drug designation for these two rare, immune-mediated conditions validates our ongoing commitment to pursuing potential first- and best-in-class medicines for diseases that affect small populations but persist with unmet medical need. Our continued exploration of rilzabrutinib across multiple indications speaks to our belief in its potential for multi-immune modulation, as well as our belief in supporting treatment options, no matter how rare a condition."*

Rilzabrutinib is currently under regulatory review in the US, the EU, and China for its potential use in immune thrombocytopenia (ITP). The target action date for the FDA regulatory decision for ITP, which was granted fast track designation, is August 29, 2025. Rilzabrutinib also received orphan drug designation for ITP in the US, EU, and Japan.

wAIHA and IgG4-RD supporting data

Results from a phase 2b study on wAIHA presented at ASH 2024 (clinical study identifier: NCT05002777) demonstrated that treatment with rilzabrutinib showed clinically meaningful outcomes on response rate and disease markers.

In IgG4-RD patients, results from a phase 2a study (clinical study identifier: NCT04520451) showed treatment with rilzabrutinib for 52 weeks led to reduction in disease flare, other disease markers, and glucocorticoid sparing. More detailed results will be shared at a forthcoming medical meeting.

The safety profile of rilzabrutinib in both studies was consistent with previous studies.

About rilzabrutinib

Rilzabrutinib is an investigational, oral, reversible, BTK inhibitor that has the potential to be a first- and best-in-class treatment of several immune-mediated diseases. BTK, expressed in B cells, macrophages, and other innate immune cells, plays a critical role in inflammatory pathways and multiple immune-mediated disease processes. With the application of Sanofi's TAILORED COVALENCY® technology, rilzabrutinib can selectively inhibit the BTK target while potentially reducing the risk of off-target side effects. Based on its ability to drive multi-immune modulation, rilzabrutinib holds great promise in the treatment of multiple clinical indications.

About wAIHA

Affecting one to three people out of 100,000 in the US each year, wAIHA is a rare, potentially life-threatening, autoimmune disorder where autoantibodies lead to the premature destruction

of the body's own red blood cells (hemolysis). People living with wAIHA may experience debilitating fatigue, thromboembolism, dizziness, palpitations, and shortness of breath as the rate of production of new red blood cells in their bone marrow cannot compensate quickly enough for premature destruction of red blood cells.

About IgG4-RD

IgG4-RD affects approximatively eight out of 100,000 adult patients in the US each year and is a rare, progressive, relapsing, chronic fibro-inflammatory condition which can manifest in almost every organ and can lead to organ damage and irreversible dysfunction with a sometimes-fatal outcome.

About ITP

ITP is a rare, complex autoimmune disorder characterized by low platelet counts (less than 100,000/µL) resulting from both increased platelet destruction and decreased platelet production. Beyond bruising and bleeding, which can include potentially life-threatening episodes like intracranial hemorrhage, people living with ITP may experience arterial or venous thrombosis, which can result from the ITP itself, from other medical comorbidities, or may also be associated with the use of certain other ITP treatments. Additionally, people living with ITP often experience easily overlooked symptoms that can significantly impair their quality of life, such as unexplained fatigue, anxiety or depression, and cognitive impairment.

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Media Relations

Sandrine Guendoul | +33 6 25 09 14 25 | sandrine.guendoul@sanofi.com
Evan Berland | +1 215 432 0234 | evan.berland@sanofi.com
Nicolas Obrist | +33 6 77 21 27 55 | nicolas.obrist@sanofi.com
Léo Le Bourhis | +33 6 75 06 43 81 | leo.lebourhis@sanofi.com
Victor Rouault | +33 6 70 93 71 40 | victor.rouault@sanofi.com
Timothy Gilbert | +1 516 521 2929 | timothy.gilbert@sanofi.com

Investor Relations

Thomas Kudsk Larsen |+44 7545 513 693 | thomas.larsen@sanofi.com
Alizé Kaisserian | +33 6 47 04 12 11 | alize.kaisserian@sanofi.com
Felix Lauscher | +1 908 612 7239 | felix.lauscher@sanofi.com
Keita Browne | +1 781 249 1766 | keita.browne@sanofi.com
Nathalie Pham | +33 7 85 93 30 17 | nathalie.pham@sanofi.com
Tarik Elgoutni | +1 617 710 3587 | tarik.elgoutni@sanofi.com
Thibaud Châtelet | +33 6 80 80 89 90 | thibaud.chatelet@sanofi.com
Yun Li | +33 6 84 00 90 72 | yun.li3@sanofi.com

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